Dystrophin minigene constructs

Human dystrophin coding sequence 11058bp

-- N-- H1 R1 R2 R3 H2 R4 R5 R6 R7 R8 R9 R10 R11 R12 R13 R14 R15 R16 R17 R18 R19 H3 R20 R21 R22 R23 R24 H4 CR CT

DysM3 (3700bp) **A2796** Δ3447 510 Δ3849 Δ3531 Δ3990 Δ4173 Δ3 -N-H1 R1 R2 R3 R22 R23 R24 H4 CR -N-H1 R1 R2 H3 R22 R23 R24 H4 CR -N-H1 R1 R2 R22 R23 R24 H4 CR -N-HIRI H4 CR N-H1 R1 R2 R23 R24 H4 CR -N-H1 R1 R22 R23 R24 H4 CR -N-H1 R1 R2 R3 R24 H4 CR -N-H1 R1 R24 H4 CR

PolyA TTR

Dystrophin minigene

ITR CMV Promoter

AAV-CMV-mini-dystrophin

AAV-MCK-mini-dystrophin

PolyA TTR

Dystrophin minigene

ITR MCK Promoter

mdx+∆3849

mdx+∆3990

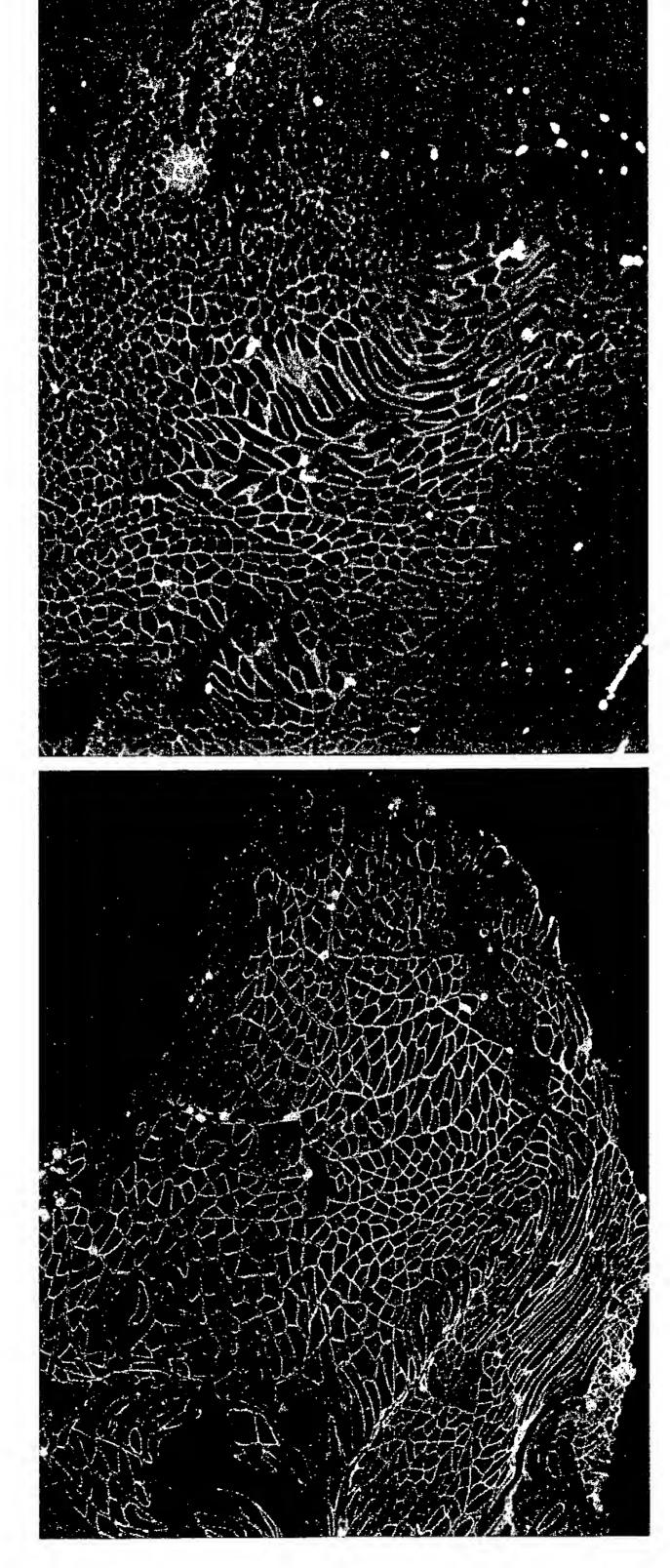


FIG. 2A

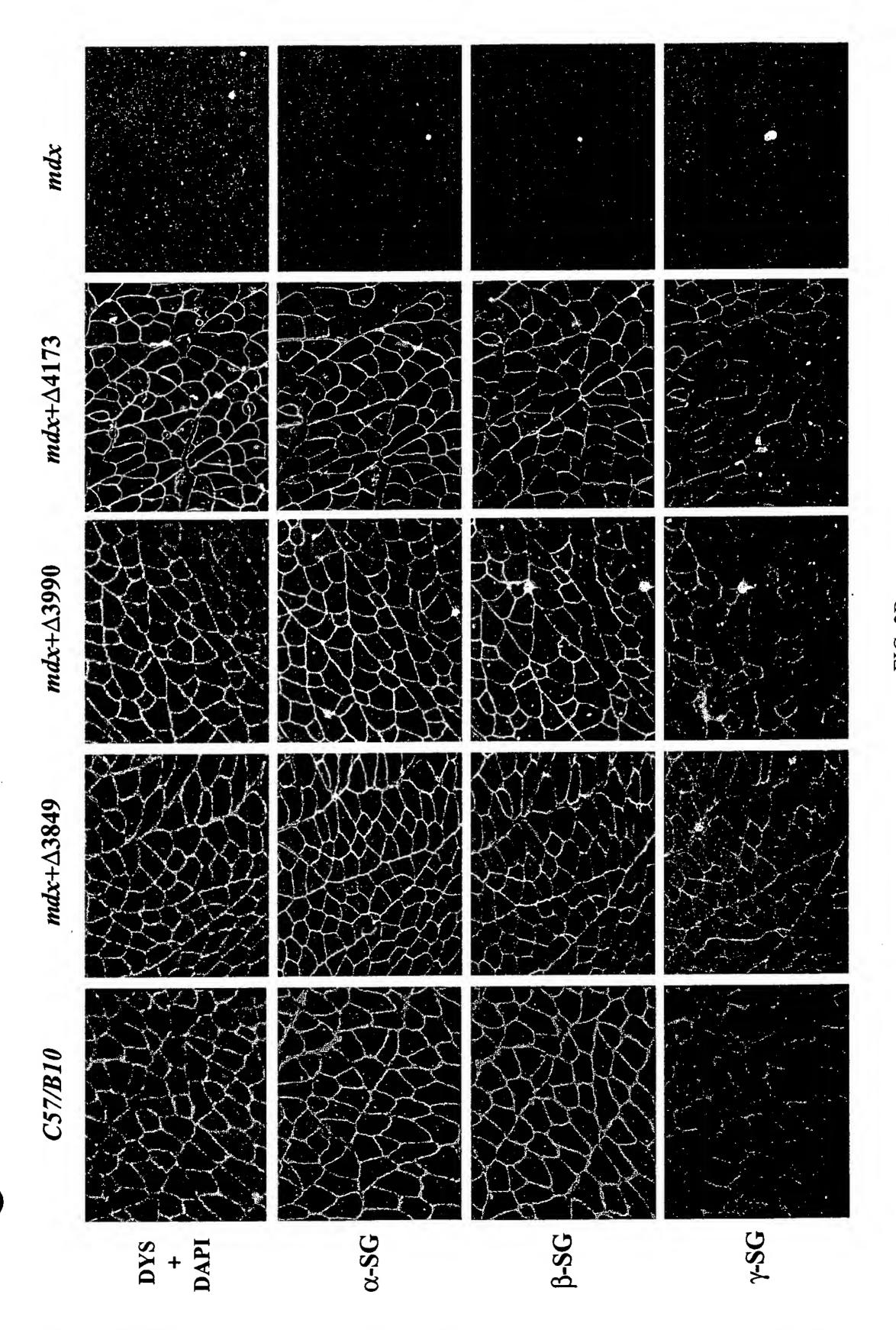


FIG. 2B

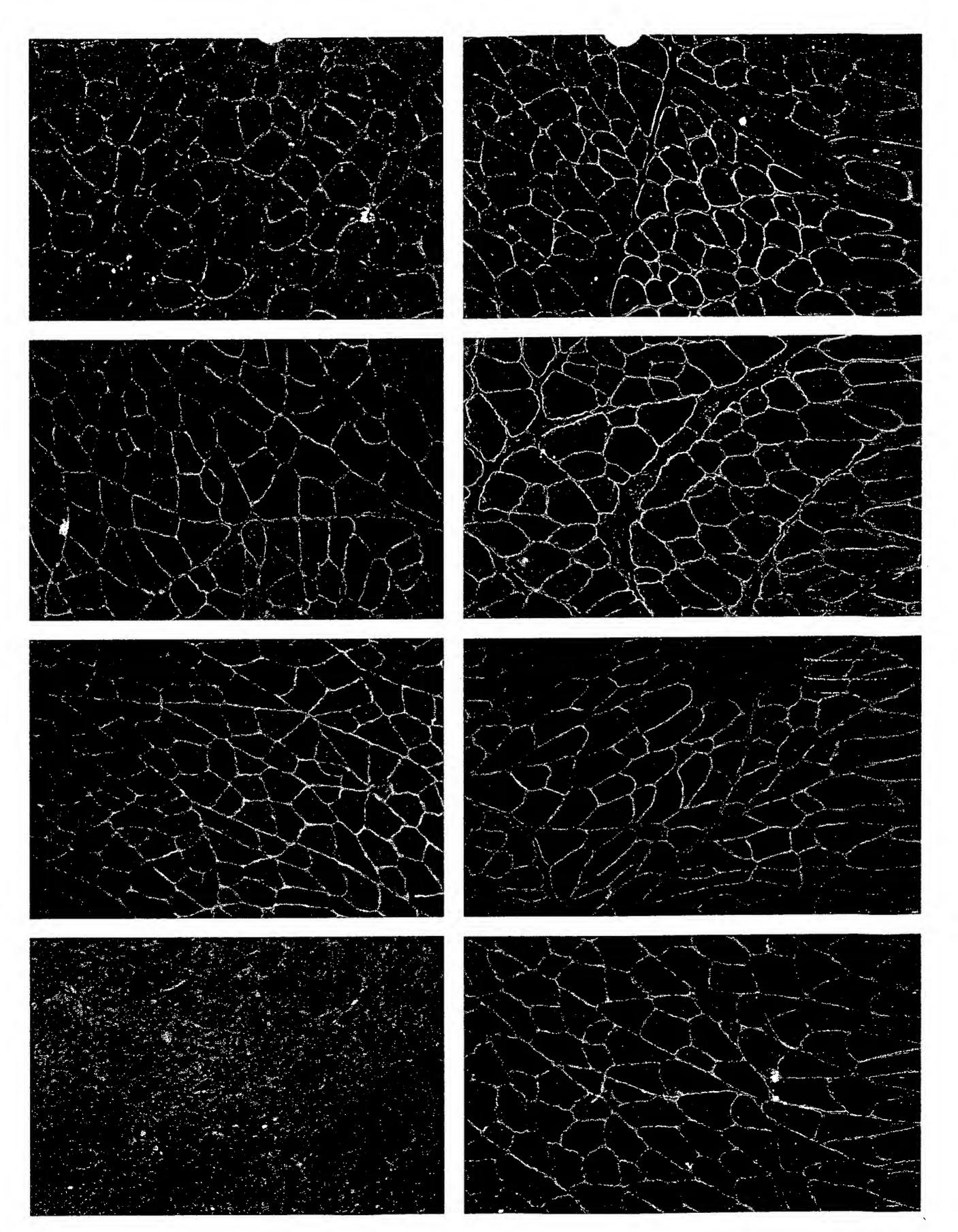


FIG. 4A

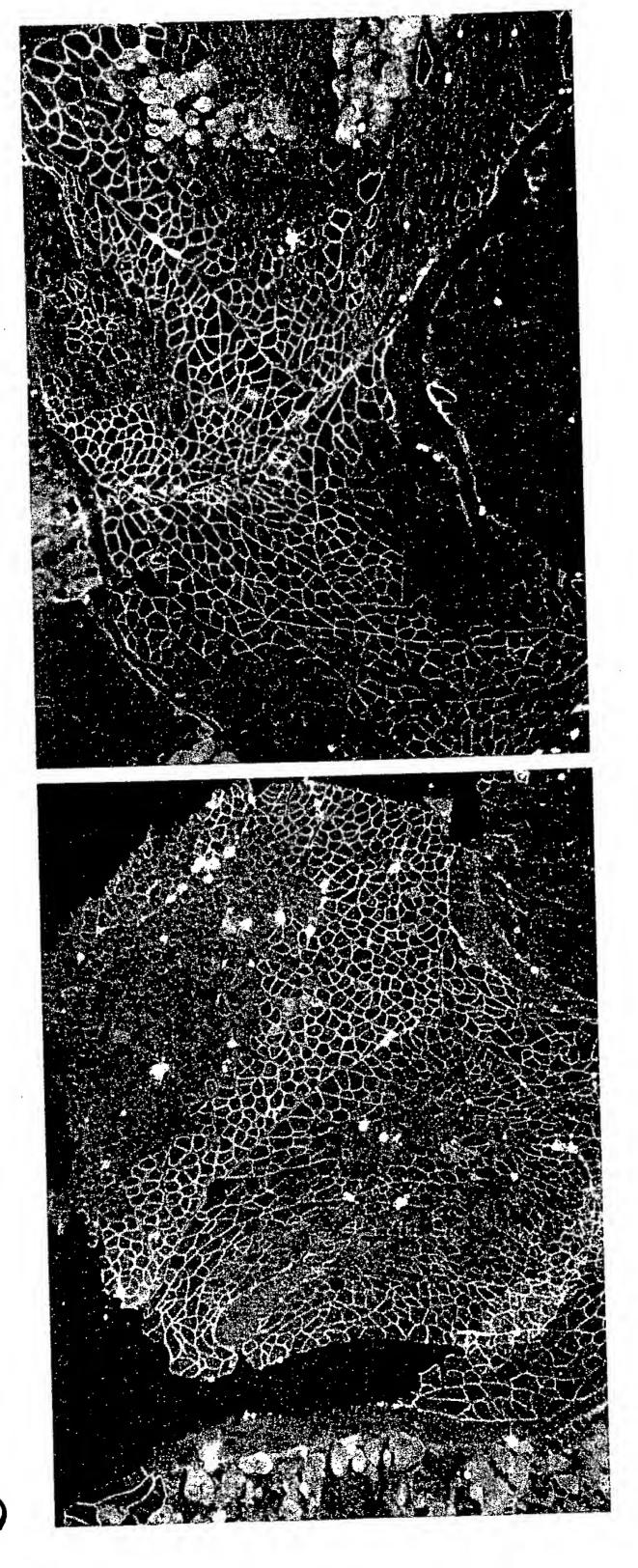


FIG. 4B

FIG. 5

Lengthening Activation

